

Congress of the United States

Washington, DC 20515

April 3, 2009

The Honorable David Obey
Chairman
Subcommittee on Labor, Health and
Human Services and Education
Committee on Appropriations
U.S. House of Representatives
2358 Rayburn HOB
Washington, DC 20515

The Honorable Todd Tiahrt
Ranking Member
Subcommittee on Labor, Health and
Human Services and Education
Committee on Appropriations
U.S. House of Representatives
2441 Rayburn HOB
Washington, DC 20515

Dear Chairman Obey and Ranking Member Tiahrt:

As Members of Congress committed to improving the health of children and adults in the United States, we respectfully request your support for the treatment and cure of Fragile X Syndrome and its related conditions. Specifically, we are seeking:

- Appropriations Report Language requesting that the National Institutes of Health:
 - Enhance its efforts across its Institutes to translate basic science findings into viable treatments for Fragile X, and encourage clinical drug trials for this orphan indication.
 - Maximize Fragile X resources by ensuring that appropriate assistance and direction are provided to implement the objectives outlined in the Fragile X Research Blueprint.
 - Strengthen and broaden research on Fragile X, Fragile X-associated tremor/ataxia syndrome (FXTAS), and Fragile X-associated primary ovarian insufficiency (FXPOI).
- \$2 million in new funding for the National Fragile X Public Health Initiative and other Centers for Disease Control and Prevention (CDC) activities such as collaboration with the Fragile X Clinical & Research Consortium to pursue initiatives that would have the greatest impact on individuals living with Fragile X across the generations.

Fragile X Syndrome is a genetic disorder that causes behavioral, developmental and language disabilities across a person's lifespan. It is linked to mutations on the X chromosome, and is the most common inherited form of intellectual disabilities. Fragile X has also been linked to early menopause (FXPOI) in female carriers and FXTAS, a Parkinson's like condition in older male carriers. Over 100,000 Americans have Fragile X Syndrome and over one-million Americans carry a Fragile X mutation and are at risk of developing a Fragile X-associated disorder.

The Subcommittee's past support for the National Institute of Child Health and Human Development-funded Fragile X Research Centers and the CDC's Fragile X Clinical & Research Consortium has had a large impact on understanding Fragile X and assisting individuals living with the Syndrome. Yet there is even more that needs to be done. In order to translate basic scientific findings into viable treatments for Fragile X, additional coordination and resources are required.

While we understand the challenges the Subcommittee faces in prioritizing requests, we believe support for enhancing Fragile X research and public health activities is imperative given the significant impact Fragile X has on families and communities across the country and the great potential for effective treatments. We thank you for your consideration.

Sincerely,



Bill Delahunt
Member of Congress



Phil Hare
Member of Congress



Gregg Harper
Member of Congress

**FRAGILE X APPROPRIATIONS REPORT LANGUAGE
SUGGESTED FY 2010**

**NATIONAL INSTITUTES OF HEALTH (NIH)
OFFICE OF THE DIRECTOR**

Fragile X - The Committee commends the National Institutes of Health for developing the NIH Research Plan on Fragile X Syndrome and Associated Disorders. The Committee congratulates NIH and its private foundation partners for providing a Small Business Research grant to fund fragile X drug development. This public-private partnership should serve as a model for future programs by small companies for fragile X and other rare diseases. We urge NIH to request proposals of this kind and to fund more of this type of innovative preclinical and clinical research. The Director is encouraged to dedicate sufficient resources to implement the NIH Research Plan on Fragile X Syndrome and Associated Disorders with the guidance of the recently established Fragile X Research Coordinating Group. The Committee expects that this initiative will be collaborative with the NICHD Fragile X Research Centers, as well as the Fragile X Clinical & Research Consortium, including support of timely clinical trials of therapies for treatment of Fragile X Syndrome and to pursue additional promising trials utilizing the collaborative network of leading clinics and research entities engaged in the Fragile X Clinical & Research Consortium. The Director is encouraged to support funding for translational research that shows significant promise of a safe and effective treatment for Fragile X Syndrome and Associated Disorders. The Committee urges the NIH, working with the Fragile X Clinical & Research Consortium, to convene a consensus conference on translational research opportunities no later than June 1, 2010.

**CENTERS FOR DISEASE CONTROL AND PREVENTION (CDC)
BIRTH DEFECTS, DEVELOPMENTAL DISABILITIES, DISABILITY AND HEALTH (NCBDDD)**

Fragile X - Within the increase for human development and disability, the Committee has included an additional \$2,000,000 over the FY 2009 level to support CDC's continuation of public health activities in the areas of Fragile X Syndrome and Associated Disorders. The Committee urges the CDC to focus its efforts on identifying ongoing needs, effective treatments and positive outcomes for families by increasing epidemiological research, surveillance, screening efforts, and the introduction of early interventions and supports for individuals living with Fragile X Syndrome and Associated Disorders. The Committee commends the CDC's current collaboration with NICHD and the newly formed Fragile X Clinical & Research Consortium. The CDC should focus funds within the Fragile X program on the continued growth and development of initiatives that support health promotion activities and foster rapid, high-impact translational research practice for the successful treatment of Fragile X Syndrome and Associated Disorders, including ongoing collaborative activities. The Committee directs the CDC to provide the Committee a progress report on all Fragile X activities by June 1, 2010.

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